THE IMPLICATIONS OF COST-EFFECTIVENESS ANALYSIS OF MEDICAL TECHNOLOGY

AUGUST 1980

Summary

CONGRESS OF THE UNITED STATES
Office of Technology Assessment
Washington, D. C. 20510

Office of Technology Assessment

Congressional Board

Representative MORRIS K. UDALL, Arizona, *Chairman*

Senator TED STEVENS, Alaska, *Vice Chairman*

Senate

EDWARD M. KENNEDY
Massachusetts

ERNEST F. HOLLINGS
South Carolina

ADLAI E. STEVENSON
Illinois

ORRIN G. HATCH
Utah

CHARLES McC. MATHIAS, JR.
Maryland

House

GEORGE E. BROWN, JR.
California

JOHN D. DINGELL
Michigan

LARRY WINN, JR.
Kansas

CLARENCE E. MILLER
Ohio

JOHN W. WYDLER
New York

JOHN H. GIBBONS
ex officio

Director's Office

JOHN H. GIBBONS, *Director*

DANIEL De SIMONE, *Deputy Director*

Advisory Council

FREDERICK C. ROBBINS
Chairman

JEROME B. WIESNER
Vice Chairman

J. FRED BUCY

CLAIRE T. DEDRICK

JAMES C. FLETCHER

GILBERT GUDE

CARL N. HODGES

CHARLES N. KIMBALL

JOHN T. McALISTER, JR.

RACHEL McCULLOCH

ELMER B. STAATS

LEWIS THOMAS

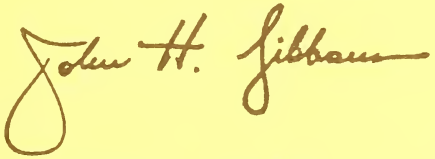
The Technology Assessment Board approves the release of this report, which identifies a range of viewpoints on a significant issue facing the U.S. Congress. The views expressed in this report are not necessarily those of the Board, OTA Advisory Council, or of individual members thereof.

Foreword

This summary presents the findings of an assessment of the use of cost-effectiveness analysis (CEA) and cost-benefit analysis (CBA) in decisionmaking regarding medical technologies. At the request of the Senate Committee on Labor and Human Resources, OTA studied the feasibility, past and potential usefulness of CEA and CBA in health care, and the implications of expanded use of these techniques.

The assessment examines the general role of CEA and CBA in health care decisions, issues related to the methodological state-of-the-art, the potential of CEA and CBA for constraining the growth in health care costs, and the past and possible future use of CEA or CBA in six specific health-related programs: medicare reimbursement, health planning, Professional Standards Review Organizations, market approval of drugs and medical devices, R&D activities, and health maintenance organizations. This summary presents the major findings and policy options related to those issues.

The full assessment includes a main report and five Background Papers. These contain: an examination of methodological issues and literature review, a case study on diagnostic X-ray, a case study on psychotherapy, 17 other case studies of specific medical technologies, and an examination of international experience in managing medical technology.



JOHN H. GIBBONS
Director

66-259 O - 80 - 2 : QL 3

Cost-Effectiveness Analysis Project Staff

Joyce C. Lashof, *Assistant Director, OTA*
Health and Life Sciences Division

H. David Banta, *Program Manager*

Clyde J. Behney, *Project Director*

Bryan R. Luce, *Senior Analyst*
Lawrence Miike, *Senior Analyst*
Michael A. Riddiough, *Senior Analyst*
Barbara Greenberg, *Senior Analyst*
Karen J. Kohlhof, *Research Associate*
Chester Strobel, *Research Associate*
Leonard Saxe, *Congressional Fellow*
Kerry Britten Kemp, *Editor and Writer*
Virginia Cwalina, *Administrative Assistant*
Shirley Ann Gayheart, *Secretary*
Nancy L. Kenney, *Secretary*

Contractors

Kenneth E. Warner, University of Michigan
The Hastings Center
Lewin and Associates
Robert A. Derzon

OTA Publishing Staff

John C. Holmes, *Publishing Officer*

Kathie S. Boss
Debra M. Datcher
Joanne Mattingly

Advisory Panel on Cost-Effectiveness Analysis of Medical Technology

John R. Hogness, *Panel Chairman*
Association of Academic Health Centers

Stuart H. Altman
Brandeis University

James L. Bennington
Children's Hospital of San Francisco

John D. Chase
University of Washington School of Medicine

Joseph Fletcher
University of Virginia

Clark C. Havighurst
Duke University

Sheldon Leonard
General Electric Co.

Barbara J. McNeil
Peter Bent Brigham Hospital

Robert H. Moser
American College of Physicians

Frederick Mosteller
Harvard University

Robert M. Sigmond
Blue Cross and Blue Shield Associations

Jane Sisk Willems
Veterans Administration

The Implications of Cost-Effectiveness Analysis of Medical Technology

Nature never gives anything to anyone; everything is sold. It is only in the abstractions of ideals that choice comes without consequences.

Ralph Waldo Emerson

The rapid and continuing growth of expenditures is a central issue in many policy decisions concerning the medical care system of the United States. Policymakers, health professionals, and consumers are seeking ways to control this growth while simultaneously improving the quality of health care. Increasingly, the use of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) is being advocated as a possible means of making the medical care system more efficient. In particular, this technique is suggested for use in health care programs—for example, by the medicare program in its reimbursement coverage decisions. Nevertheless, a great deal of confusion and disagreement surrounds the implications and feasibility of applying CEA/CBA in health care. To aid in their decisions concerning the possible use of CEA/CBA in Federal health programs, the Senate Committees on Labor and Human Resources and on Finance asked OTA to explore the applicability of CEA/CBA to medical technology.

In the assessment, three major issues are examined: 1) the general value of CEA/CBA in decisionmaking about the use of medical technology; 2) the methodological strengths and shortcomings of the techniques; and 3) the potential for initiating or expanding the use of CEA/CBA in six health care programs—reimbursement coverage, the Professional Standards Review Organizations (PSROs), health planning, market approval for drugs and medical devices, R&D activities, and health maintenance organizations (HMOs)—and, most importantly, the implications of any expanded use.

The prime focus of the assessment is on the application of CEA/CBA to medical technology, i.e., the drugs, devices, medical and surgical procedures used in medical care, and the organizational and support systems within which such care is provided. Except in a background paper on psychotherapy, the report does not address psychosocial medicine. Other factors influencing health, such as the environment, are not directly covered either. The findings of this assessment, though, might very well apply to health care resource decisionmaking in general, and with modification, to other policy areas such as education, the environment, and occupational safety and health.

This OTA assessment finds that CEA/CBA cannot serve as the sole or primary determinant of a health care decision. Decisionmaking could be improved, however, by the process of identifying and considering all the relevant costs and benefits of a decision. At present, using the approach or *process* of CEA/CBA in decisionmaking may be more helpful than the rigid and formal application of CEA/CBA *study results* in health care program decisions. It is unrealistic, moreover, to expect that CEA/CBA, in itself, would be an effective tool for reducing or controlling overall expenditures for medical care.

EVALUATING COSTS AND BENEFITS

All decisions have consequences. Usually, however, in most decisionmaking processes only a fraction of all potential consequences is taken into account. The inherent complexities and uncertainties associated with many decisions make it extremely difficult to identify and weigh all possible consequences. In general, however, the quality or validity of decisions can be increased by analysis that forces a structuring of the decision process—that provides a consistent framework for identifying and considering as many of the relevant costs and benefits as is feasible.

The public, or governmental, sector is called upon to make certain decisions that are impractical for the private sector to make. Examples of these are decisions concerning national defense or air pollution control, neither of which is amenable to being traded in the marketplace. Other decisions are made by the public sector for social reasons such as assuring equitable distribution of what may be considered essential goods (e.g., health care for the elderly). Because conventional private sector techniques, such as capital budgeting and return-on-investment analysis, are insufficient for these

decisions, techniques such as CBA and CEA have been developed. In the medical care area, CEA/CBA is designed to integrate the economic aspects of a decision with the health aspects of that decision. Consequently, **it should not be considered simply an economic tool.**

There are two basic types of health care resource allocation decisions which *in theory* could benefit from a CEA/CBA process. The first are decisions made within a fixed or prospectively set budget, such as those made by HMOs. The second are decisions made in the absence of a direct budget constraint, such as those made for medicare reimbursement or in health planning.

In the former—allocation decisions made within a budget—tradeoffs must be made, since not all projects can be funded. The projects that promise to deliver more benefits for the cost should be more attractive than those projects expected to deliver fewer benefits. In these decisions, the functions of CEA/CBA would be to illuminate the decision process and to require that implicit judgments be made more explicitly, thus allowing external examination of the assumptions and values placed on decision variables.

In nonconstrained decisions, direct tradeoffs between competing projects often are unnecessary. Consequently, a function of CEA/CBA in these decisions would be to force consideration of economic factors. In health planning decisions, for example, planners would be asked to consider not only whether a service is needed but also whether it is worth the cost.

COST-BENEFIT ANALYSIS AND COST-EFFECTIVENESS ANALYSIS

The terms CBA and CEA refer to formal analytical techniques for comparing the positive and negative consequences of alternative ways to allocate resources. In practice, the comparison of costs and benefits is accomplished through various analytical approaches which comprise a spectrum ranging from sophisticated computer-based analysis using large amounts of epidemiological and other data to partially intuitive, best-guess estimates of costs and benefits. Some analyses may take into account the results of clinical trials of a technology and model the technology's effect on health outcomes. Others may assume that the alternative technologies under study have equal effectiveness and concentrate on the difference in costs involved.

Thus, there is a continuum of analyses that examine costs and benefits. At one end of the continuum are what will be referred to as "net cost" studies. In these studies the emphasis is on costs, and net cost studies in the past have often assumed benefits or efficacy to be equal. At the other end of the continuum are analyses that attempt to relate the use of the technologies under study to specific health-related outcomes and to compare the costs of the technologies to the differences in health benefits. CBA and CEA comprise the entire set of analytical techniques—differentiated by the specifics of what costs and benefits are considered and how they are analyzed—on this continuum.

The principal distinctions between CEA and CBA lie in the valuation of the desirable consequences of a decision, in the implications of the different methods of that valuation, and usually in the scope of the analysis. In CBA, all costs and all benefits are valued in monetary terms. Thus, conceptually, CBA can be used to evaluate the "worth" of a project and would allow comparison of projects of different types (such as dams and hospitals). In CEA, the health-related effects of programs or technologies are not valued in monetary terms but rather are measured in some other unit (such as years of life gained). A CEA, therefore, does not result in a net monetary value for a project. Instead, it produces a measure of the cost involved in attaining some desirable health-related effect. Conceptually, CEA permits direct comparison of only those programs or technologies that share similar objectives. This OTA assessment uses the term CEA/CBA to refer to both of these techniques because the findings below apply generally to both.

FINDINGS

General

Most of the specific findings presented below relate to the two major, general findings of the OTA assessment:

- Performing an analysis of costs and benefits can be very helpful to decisionmakers because **the process of analysis gives structure to the problem, allows an open consideration of all relevant effects of a decision, and forces the explicit treatment of key assumptions.**
- CEA/CBA exhibits **too many methodological and other limitations, however, to justify relying solely on the results of formal CEA/CBA studies in making a decision. Thus, although CEA/CBA is useful for assisting in many decisions, it should not be the sole or prime determinant of a decision.**

CEA/CBA is viewed by different parties as ranging in usefulness from obfuscating the pertinent issues in a decision process at one extreme to illuminating and synthesizing these issues so well that the technique can be used to make decisions at the other extreme. There is, however, a middle position which maintains that **the technique could be helpful in structuring information and that this information should be only one of several components of a decision process.** The OTA case study on the artificial heart, for example, lays out many of the factors to be considered in decisions regarding funding of R&D of biomedical technologies. But those decisions will also be dependent on a number of other political and social factors that are difficult to analyze systematically.

Both extreme positions mentioned above pertain to the use of CEA/CBA as a formal, structured analysis which is oriented toward a bottom-line answer (e.g., a cost-benefit ratio). Such a bottom line, however, often hides many important value judgments, thus providing a seemingly unambiguous answer that may rest on ambiguous data or assumptions.

Advocates of the middle position propose that CEA/CBA be used within the context of accepted principles of analysis in order to illuminate the costs and the benefits of a decision but not necessarily to aggregate and weigh them.

The findings of this and any other assessments of the past usefulness or current potential of CEA/CBA in health care decisionmaking should be kept in perspective: Because there has been little experience with the use of CEA/CBA, these findings are based on very little definite information. Therefore, any of the findings or projections of the usefulness or implications of CEA/CBA could be proven wrong as more experience accumulates. In fact, one of the priorities for future examinations of the role of CEA/CBA in health care should perhaps be small, controlled experiments, or demonstrations, of its potential use (see Option 10 below).

Methodology

There is no set combination of specific analytical elements that form a standard CEA or CBA method. A standard or rigid method, however, is to be neither expected nor desired. OTA found a wide variation in the forms of CEA/CBA analyses actually conducted to date. Most of the analyses reviewed seem to be academic exercises, infrequently connected to the policy process. In addition, the literature on CEA/CBA reveals that a great many of the analyses conducted tend to be on the "net cost" end of the CEA/CBA continu-

um, that is, analyses in which health outcomes were not taken into explicit account. In part, this may be a reflection of the paucity of data on the efficacy, safety, and appropriate use of medical technology.

OTA's assessment does find, however, general agreement on a set of 10 principles of analysis that could be used to guide the conduct, evaluation, or use of CEA/CBA studies. OTA believes that use of generally accepted principles is important, largely because of the basic methodological limitations of CEA/CBA. One limitation, for example, is that although the results of a CEA/CBA often are dependent on the discount rate chosen, there is no general agreement on what discount rate should be used under what circumstances. Furthermore, as primarily an efficiency-oriented technique, CEA/CBA is weak in the areas of equity and other ethical considerations. Finally, CEA/CBA must often address issues of great uncertainty, such as disease progression, patient compliance rates, differing responses to technology by differing population groups, and so on. These and other limitations must be kept in mind and must to the maximum extent feasible be compensated for by techniques such as sensitivity analysis (see #8 below). The 10 basic principles of analysis are:

1. **Define Problem.**—The problem should be clearly and explicitly defined and the relationship to health outcome or status should be stated.
2. **State Objectives.**—The objectives of the technology being assessed should be explicitly stated, and the analysis should address the degree to which the objectives are (expected to be) met.
3. **Identify Alternatives.**—Alternative means (technologies) to accomplish the objectives should be identified and subjected

Ten General Principles of Analysis (for CEA/CBA methodology)

1. Define problem
2. State objectives
3. Identify alternatives
4. Analyze benefits/effects
5. Analyze costs
6. Differentiate perspective of analysis
7. Perform discounting
8. Analyze uncertainties
9. Address ethical issues
10. Interpret results

SOURCE: Office of Technology Assessment.

to analysis. When slightly different outcomes are involved, the effect this difference will have on the analysis should be examined.

4. **Analyze Benefits/Effects.**—All foreseeable benefits/effects (positive and negative outcomes) should be identified, and when possible, should be measured. When possible, and if agreement on the terms can be reached, it may be helpful to value all benefits in common terms in order to make comparisons easier.
5. **Analyze Costs.**—All expected costs should be identified, and when possible, should be measured and valued in dollars.
6. **Differentiate Perspective of Analysis.**—When private or program benefits and costs differ from social benefits and costs (and if a private or program perspective is appropriate for the analysis), the differences should be identified.
7. **Perform Discounting.**—All future costs and benefits should be discounted to their present value.
8. **Analyze Uncertainties.**—Sensitivity analysis should be conducted. Key variables should be analyzed to determine the importance of their uncertainty to the results of the analysis. A range of possible values for each variable should be examined for effects on results.
9. **Address Ethical Issues.**—Ethical issues should be identified, discussed, and placed in appropriate perspective relative to the rest of the analysis and the objectives of the technology.
10. **Discuss Results.**—The results of the analysis should be discussed in terms of validity, sensitivity to changes in assumptions, and implications for policy or decisionmaking.

In addition to conforming to these 10 principles, all quantitative analyses should specify data sources and be written as clearly and nontechnically as possible. They should also be subjected to review—including public scrutiny when appropriate—especially regarding assumptions upon which their outcomes may rest.

OTA found that many of the methodological limitations of CEA/CBA are often hidden by the practice of deriving a cost-benefit or cost-effectiveness ratio—that is, a numerical bottom-line. Therefore, **the possibility of not aggregating the often complex sets of calculations should be investigated.** Instead of aggregating, analyses might be done by explicitly listing or ARRAYING all the elements which are included in or would be affected by a decision. Where costs and effects could be quantified, that would be done; where they could be combined, that would be done. But no effort would be made to arrive at a single combined value when one or

66-259 O - 80 - 3 : QL 3

more important nonquantifiable variables would have to be left out or relegated to a footnote. An array method of analysis would mean that decisionmakers would have a greater number of elements to consider, but it would also mean that intangible or nonquantifiable factors would be more explicit and, thus, more likely to be taken into consideration.

The findings of this assessment, especially methodological ones, focus primarily on the practical implications of CEA/CBA for health policy. The report is not written for the academic research community. The general principles above apply to analysis for policy use. In no way, however, should the findings be taken to mean that complex, sophisticated analysis is always unnecessary. Advancement of the state-of-the-art of CEA/CBA requires ongoing and sophisticated research. Current research on the development and validation of sets of indexes of the health status of a population, for example, appears very valuable. Use of such complex aspects of CEA/CBA, however, may require greater technical capabilities than most health programs currently possess. Addressing a more limited approach to analysis, then, seems appropriate for the goal of this assessment. But it does not diminish the need for more complex approaches in research or other specialized circumstances.

Applying CEA/CBA in Health Care

As stated above, CEA/CBA can be more valuable to health care decisionmaking when it serves as a problem structuring process than when it becomes the primary factor, with numerical results, of a decision. **Furthermore, CEA/CBA potentially can be more valuable for decisionmaking under a constrained budget, when tradeoffs have to be made directly, than when constraints are nonexistent or very indirect. In neither case, however, would CEA/CBA necessarily function as an effective cost-constraining mechanism or tool.** Under the budget system, the budget itself would be the constraining mechanism. Under the nonconstrained system, since no direct tradeoffs are required, no direct limit on expenditures is set. CEA/CBA might, however, change the mix of expenditures. Especially under a budget system, technologies might be substituted for one another as a result of analysis.

The context within which a decision will take place must be specified before any judgment of the usefulness or applicability of CEA/CBA can be made. For example, does the decision relate to a technology at an early stage in its lifecycle such as bone marrow transplantation? Or does it concern an established technology,

such as cervical cancer screening? Is the technology in question a diagnostic technology, such as the CT scanner, or a therapeutic one, such as renal dialysis? The possibility of affecting the course of a technology's diffusion and use might be greater in early stages of its development, but the uncertainties about its health effects and its costs will generally be greater. Thus, it may be possible to do a more valid or certain CEA/CBA later in the technology's lifecycle, but the information gained may be less valuable for public policy. The tradeoff required will vary depending on the specifics of the technology and the policy decision to be made. Similarly, diagnostic technologies are often more difficult to study than other types because of the uncertainties involved in linking their use to health outcomes. Thus, **studies of diagnostic technologies often tend toward the "net cost" end of the CEA/CBA spectrum, where the measures of outcome or benefit may be numbers of tests performed or levels of diagnostic accuracy.**

Applying CEA/CBA in Specific Health Care Programs

OTA examined the current and potential use of CEA/CBA or related techniques in six health care programs. Although informal and often implicit analysis of costs and benefits frequently takes place in health policy formulation, OTA found very little formal use of CEA/CBA in the programs studied. In several of the areas, cost itself has played little or no role in policy decisions.

Reimbursement programs such as medicare and Blue Cross/Blue Shield, when deciding what technologies will be covered, concentrate on criteria that generally do not explicitly include costs (e.g., stage of development of the technology and acceptance by the medical community). Under medicare, initial responsibility for identifying questions about whether a technology should be covered lies with a system of local contractors who administer the program. When not resolved at the local level, a question is referred to the Health Care Financing Administration (HCFA), which may seek a recommendation from the Public Health Service (PHS). PHS has traditionally used four criteria in its recommendations: efficacy, safety, stage of development, and acceptance by the medical community. Other health insurance programs, such as Blue Cross/Blue Shield, operate similarly.

The possibility of expanding coverage criteria to explicitly include costs or cost effectiveness is being examined by HCFA and PHS. The first question to be answered is whether there is a legal basis for any such inclusion. Currently, the language of the Social Security Act requires the medicare program to pay for services that

are "reasonable and necessary." There is no definitive interpretation of whether that language means that the relative "cost effectiveness" of a particular technology might make it unreasonable or unnecessary.

Most current reimbursement programs are examples of programs without direct budget constraints. Each reimbursement coverage decision is not seen as involving a tradeoff (though in broader terms it certainly involves one). Approval of one technology does not mean that another will not be covered. In a very real sense, **the existing reimbursement system is an open-ended system of financing medical care.**

The **PSRO program** was enacted to assure that health services provided under medicare, medicaid, and certain other programs are medically necessary, meet professionally recognized standards of care, and are provided at the most economical level consistent with quality care. CEA/CBA approaches have theoretical applicability in three areas: 1) the development of standards of care against which actual practices are judged, 2) the internal management of individual PSROs, and 3) evaluations of the national PSRO effort. Cost-effectiveness criteria have not been directly incorporated into standards of care except in a few instances, but it appears possible for PSROs to do so, although the studies would most likely have to be conducted elsewhere. PSROs generally do not have the analytical capabilities for CEA/CBA. Net cost techniques have been used to evaluate whether the savings achieved outweigh the costs of administering the review activities of the overall PSRO program. These analyses, which often reach contradictory conclusions, do not, however, examine costs in relation to changes in health outcomes that may result from PSRO reviews. Nor do they address the fact that even the most optimistic reports of savings represent an infinitesimal portion of total medicare and medicaid expenditures.

Because individual PSROs operate under a program management budget, incentives exist for them to use cost-effectiveness-like approaches in choosing areas in which to concentrate their review activities.

In contrast to PSROs, in the area of **health planning,** the National Health Planning and Resources Development Act, with its amendments, explicitly states that resources are to be allocated in a more efficient manner and that health planners should weigh both costs and benefits in their decision processes. The Health Resources Administration (HRA) is emphasizing a more analytical approach to health planning, especially in regard to capital budgeting. OTA found that State health planning and development agencies

(SHPDAs) and health systems agencies (HSAs) are for the most part still primarily health "needs" oriented. An OTA survey of planning agencies indicated that few agencies, even those in the vanguard of using economic analysis for allocation of resources, are going beyond the practice of considering only capital costs. There is a slight trend, however, for the most analytically advanced staffs to consider the marginal (or incremental) costs associated with changes in use of a technology. The analysis that took place around the CT scanner is a good example of that. OTA discovered no HSAs that explicitly balance costs with health benefits in, for example, certificate-of-need recommendations. Thus, although there appear to be no legal barriers to its use, CEA/CBA has not been much applied. In health planning, as in reimbursement, there is no direct budget constraint, i.e., the area served by an HSA is not operating with a fixed or predetermined amount of resources to be spent on health care. Few pressures, therefore, act on planning agencies to force consideration of how to get the most health effect for the fewest dollars.

Market approval for drugs and medical devices, by the Food and Drug Administration (FDA), is an area where Congress has specified the decision criteria, which explicitly do not include costs. FDA is required to regulate the market introduction of drugs and devices on the basis of effectiveness (efficacy) and safety. FDA has not formally used cost-effectiveness or any other economic criteria to evaluate drug or device applications. The primary purpose of FDA is to protect the public from unsafe and inefficacious products. Although the Agency's processes do have an indirect influence on the way resources are allocated, there are several factors arguing against the incorporation of explicit economic criteria in its decision processes. Perhaps the most important of these is that by incorporating such criteria, FDA might be extending its regulatory functions beyond those envisioned by Congress. Further, the administrative burden and demands of doing so would be enormous.

The **Federal health R&D effort** encompasses a broad spectrum of activities and involves many Federal agencies. At one end of the spectrum is biomedical and behavioral research which is supported by, for example, the National Institutes of Health, and at the other end is health services research such as that supported by the National Center for Health Services Research, the National Center for Health Statistics, and HFCA. Lying somewhere in the middle of the spectrum and incorporating both end points is research supported by the National Center for Health Care Technology. The Federal agencies involved in health R&D rarely use explicit CEA/CBA considerations to set research priorities, to allocate research resources,

or to evaluate the results of research. The uncertain end products of much research makes it difficult to conduct CEA/CBA. Technologies being assessed or to be developed are often at an early stage of the lifecycle. A CEA/CBA-like approach, with no requirement for aggregation of variables, may be more applicable for R&D than is rigid, traditional CEA/CBA. The more basic the level of research, the less applicable the techniques become, owing to the increased uncertainties. Ironically, it may frequently be more desirable to assess a technology, including its cost implications, earlier in its development rather than later.

HMOs are both insurers and providers of medical care. They are an example of a program operating under a constrained budget and have a direct economic incentive to provide "cost-effective" care. However, a preliminary OTA examination of HMOs' decisionmaking criteria indicates that, at least in part because HMOs operate in a predominantly fee-for-service environment and must compete for enrollees, these organizations do not commonly weigh health benefit against cost in deciding what medical services to offer. The actual analytical approach used by HMOs seems to be related to "net cost" techniques. Although HMOs do not always consider health benefits, their use of a "net cost" approach suggests that they view the provision of technology in terms of efficiency. They seek ways to provide benefits comparable to fee-for-service medicine at the lowest cost feasible. The current potential for use of CEA/CBA by HMOs, weighing health benefits against costs, does not appear to be as large as the existence of direct budget constraints would predict. That situation may change, however, as more experience is gained with CEA/CBA and as HMOs increasingly encounter competition with each other in addition to fee-for-service health care providers.

POLICY OPTIONS

Options for congressional consideration fall into two categories: 1) those that relate to the current possibilities for using CEA/CBA in policy formulation and decisionmaking (Options 1 through 6), and 2) those that relate to the development of CEA/CBA techniques in themselves (Options 7 through 10).

Options Relating to Current Possibilities for Use of CEA/CBA

Options 1 through 6 follow a four-stage progression of confidence in the current applicability of CEA/CBA to health care programs—from a perception that its use should be discouraged (Option 1), to a neutral, status quo attitude (Option 2), to cautious encouragement (Option 3), to a positive belief that CEA/CBA can be effectively applied (Options 4 through 6). Options 1 through 3 present general approaches that are mutually exclusive for any given program area. Each of Options 4 through 6, however, relates to a specific program area.

OPTION 1

Discourage or prohibit the explicit inclusion of cost-effectiveness criteria in specified health care resource allocation decisions.

The implicit weighing of the costs and benefits of resource allocation decisions cannot—and should not—be legislated out of the policy process. It is inherently a part of that process. What this option does, therefore, is signal that the techniques available to *formalize* the now informal process of weighing these costs and benefits are not regarded as sufficiently valid to justify their use.

For those types of decisions where costs have not previously been explicitly considered to any significant extent (such as medicare coverage of specific technologies), this option would reflect a positive decision *not* to include costs explicitly. For decisions made within a constrained budget, where the costs of alternative allocations of resources are automatically, though often implicitly, factored in, this option would be a statement that existing methods of considering costs are either adequate or would not be improved on by mandatory use of formal analytical techniques.

Reflected in this option are a recognition of both the inherent methodological limitations of CEA/CBA and the weaknesses that result from the fact that CEA/CBA is still an evolving methodology; a belief that other factors in the decisionmaking context are more important than CEA/CBA-derived information; and a desire to avoid the possible misuse of CEA/CBA. These considerations, along with considerations of the country's limited experience with making health care allocation decisions based on CEA/CBA, and the expense associated with conducting large numbers of CEA/CBAs required for decisionmaking, all support this option.

However, if the health care system is in fact operating in an era of limitations on resources and if the concern over rising health care costs continues to lead to pressures for better balancing of costs with outcomes in the use of medical technology, this option becomes much less attractive. It might eliminate much of the current and future developmental work on techniques for comparing costs to benefits. It would force reliance on current methods of making decisions. If Congress is not satisfied with the allocation and use of medical technology and other health care resources and not satisfied with the cost implications of such allocations, some method for more explicitly forcing a weighing of costs with benefits may need to be developed. (Options 7 through 10 below could be a step in that direction.)

Thus, this option should not be adopted if Congress believes that more explicit balancing of costs and benefits is necessary and that CEA/CBA or similar techniques hold the potential to contribute to that process. Rejection of this option does not mean that immediate mandatory use of CEA/CBA should take place; it simply does not shut the door to that possibility *or* to the possibility of encouraging methodological development, experiments with increased use of CEA/CBA, and increased use of formal analytical techniques for balancing costs with benefits.

OPTION 2

Allow current trends in development and use of CEA/CBA to continue, but make no changes in legislative requirements or prohibitions relating to the use of CEA/CBA.

This is basically a status quo option, but it recognizes that the field of CEA/CBA is undergoing methodological change and that analysts and policymakers are paying increasing attention to the potential uses of the techniques. These trends would continue under this option. (They could be encouraged through implementation of any or all of Options 7 through 10.) But no changes in legislative language regarding the formal, explicit use of CEA/CBA in program decisions would be made. Since OTA found that very little formal use of CEA/CBA currently takes place, that nonuse would most likely continue into the near future.

Under this option, health care programs would be allowed to examine the possibility of using more explicit costs and benefits information. But they would not be required or encouraged to do so by Congress.

In medicare reimbursement decisions, this option would enable HCFA and PHS to continue their investigation into the possibility of adding cost effectiveness or some other consideration of costs to the list of coverage criteria. At the same time, the option recognizes that the negative aspects of adding cost effectiveness as a criterion (discussed below under Option 4) are quite serious. It would allow demonstration projects (as presented in Option 10) to take place before a general decision to change current reimbursement policies is made.

Current legislative language for market approval of drugs and medical devices would continue to specify only safety and effectiveness as primary decision criteria. This situation seems to reflect congressional intent to date and would avoid the problems associated with changing the laws to include cost effectiveness (as examined in Option 6 below.)

Similarly, other health care programs would be allowed to continue investigating the possibility of using more explicit costs and benefits information. But they would not be required or encouraged to do so by Congress. For example, the health planning program could continue to provide technical assistance regarding cost and cost-effectiveness analysis to local HSAs. Also, Congress could encourage or discourage the establishment and expansion of HMOs without mandating or prohibiting their use of cost-effectiveness criteria for decisionmaking regarding technology.

Selection of this option would in part reflect the view that the pros and cons of the other options are too uncertain to permit a definite decision on prohibition or on active encouragement of use of CEA/CBA. But it also would reflect a belief that some method of more explicit consideration of costs and benefits is needed.

OPTION 3

Encourage the explicit consideration of costs and benefits in resource allocation, but do not require it as a formal decision criterion.

Selection of this option requires a more favorable view of the current potential of formal analysis than does the previous option. This option, in effect, says that health care is at the point where costs should be explicitly compared to benefits when decisions about resource allocation are made, and that the techniques that are available to make such comparisons are useful—though not well enough developed to be used as mandatory aspects of the decisions. This option goes beyond the status quo. Congress, by adopt-

ing this approach in any of the decision areas, would be explicitly approving the approach of CEA/CBA. Decision areas such as health planning would receive clear signals that decisions on resource allocation should be *in part* based on formal identification and (where possible) measurement and valuation of relevant costs and benefits.

Should this approach be taken, Congress could, for example, inform HCFA and PHS that it approves of efforts examining the possibility of including cost effectiveness of medical technology as a criterion for reimbursement coverage and that it approves use of such a criterion if that examination finds it to be feasible. Reimbursement coverage is mentioned here only as an example; the difficulties in this area are substantial (see Option 4) and this may not be an area where Congress wishes to encourage CEA/CBA. Similar statements can be made in regard to encouraging the use of CEA/CBA in market approval decisions by FDA.

There are, however, areas where more explicit consideration of costs and benefits might be encouraged with less negative impact. One is in resource allocation decisions by health planning agencies. Costs and benefits are already listed as criteria for such decisions. Also, quality assurance activities of PSROs might be modified to take costs and benefits into more explicit consideration. Encouragement in these areas might take the form of providing increased analytical capabilities to agency staffs, providing technical assistance based on modified CEA/CBA-derived national data for agency use, and, importantly, signaling a congressional desire that *all* relevant costs and benefits of agency decisions be considered, not just those costs or benefits that may be easily quantified.

The essence of this option is that costs and benefits should be a *part* of resource allocation decisions, but that current methods argue against leaning on formal analysis too heavily.

A positive aspect of this option is its direct recognition of the necessity of having some way of making resource tradeoffs. The option may also result in an increase in knowledge about the value of current and, eventually, improved analytical techniques such as CEA/CBA. A negative aspect is the difficulty of encouraging use of CEA/CBA while not requiring it. Substantial thought should go into the specific means of implementing this option in individual health care programs.

OPTION 4

Specify that medicare reimbursement criteria should include consideration of technology's cost effectiveness.

This option would add consideration of costs, which have not been an explicit or direct criterion to date, to the largest Federal health program. The option's desirability depends on philosophical, methodological, and logistical factors. Philosophically, selection of this option would signal a change in the rationale of medicare and perhaps other Government-funded health programs. Currently, medicare operates, with some exceptions, as a program to enable the aged and disabled to have access to the medical services available to the general population. For some very expensive life-prolonging technologies (e.g., renal dialysis), however, medicare has become the vehicle by which those technologies are made generally available to all age groups.

If, under this option, cost-effectiveness criteria are applied to all technologies to be covered under medicare, the aged and disabled might be denied reimbursement for use of technologies that were still available to other insured populations.

Further, major changes in the procedures for raising coverage questions would have to be made. The local contractors might have to refer a great many questions to HCFA for resolution, and HCFA would depend on PHS for a large volume of analysis. Moreover, the data on which coverage recommendations and decisions would have to be made do not exist for many technologies. The administrative expense would be large and the analytical base would have to be greatly expanded at considerable cost. The potential cost savings from more cost-effective use of technologies, if that were brought about by application of CEA/CBA, would have to be compared to the expenses generated by this option. There is, however, no way to estimate confidently either the probability or the amount of medicare cost savings that might result. This option, if implemented in the absence of an overall system to control expenditures, would have little effect by itself on the absolute amount of expenditures.

Consideration might be given, however, to requiring that the possible addition of major high-expense technologies or inexpensive but high-volume technologies to medicare be carefully assessed and their potential impact on the total health budget be analyzed. Tradeoffs between increases in medicare expenses and increases in medicaid benefits or PHS programs could then be considered.

OPTION 5

Require that resource allocation decisions by health planning agencies be based in part on formal cost-effectiveness criteria.

This option implies a belief that the state-of-the-art of CEA/CBA is sufficiently advanced to provide useful and valid information to health planners. It further implies that State and local health planning communities and their constituents will accept the results of CEA/CBA studies as inputs to the decision process. The feasibility of the option also depends on health planning agencies' developing the capability to perform or commission adequate analyses or having access to studies done by others that will be adaptable to the local situations.

OTA finds that, although recent legislation does indicate that Congress intends that health benefits be weighed with costs in planning decisions and that HRA is providing some guidance to State and local planning agencies in this regard, there is no consensus as to an adequate analytical method to which health planners can turn. At the same time, however, OTA finds that helpful information for many decisions can be obtained by following generally accepted principles of analysis, which include explicitly enumerating all the costs and benefits of a given course of action. In general, health planning agencies at present do not have sufficient technical skills to perform formal CEA-type studies.

OPTION 6

Modify the food and drug laws to include cost effectiveness as a market approval criterion for drugs and medical devices.

This option might force FDA to formally compare the safety, efficacy, and costs of a new product with those same characteristics of existing technologies. Such an effort would require an extensive amount of data, much of which does not currently exist. The analytical capabilities of FDA would have to expand and change markedly to incorporate the new criterion. The administrative and analytical demands of this option would be enormous.

Most importantly, the decision of whether to select this option would hinge on current congressional intent regarding market approval. Past intent, as reflected in the statutes, explicitly mentions only safety and effectiveness (efficacy). Approving this option would mean that the intent of Congress regarding FDA's regulatory function has changed. If such is not the case, the option is inadvisable. If intent has changed, then the feasibility of the option depends on the minimization of the administrative and methodological problems.

Options Addressing the Techniques of CEA/CBA Themselves, or Their Development

OPTION 7

Encourage research on the methods of CEA and CBA, concentrating on general principles of analysis.

OPTION 8

Encourage the conduct of increased numbers of CEA/CBAs of medical technology.

OPTION 9

Encourage the development of a strategy for identifying and collecting information needed for CEA/CBA.

OPTION 10

Require or encourage demonstration projects to test the feasibility of CEA/CBA as an aid in decisionmaking.

These options are not mutually exclusive; any combination of them could be implemented. They are designed to yield information about the future usefulness of CEA/CBA in decisionmaking and to increase CEA/CBA's potential for being useful. None of the options requires an immediate commitment to or decision on the ultimate use of CEA/CBA. All might contribute to advancement of the state-of-the-art of CEA/CBA.

Research as proposed in *Option 7* might concentrate on issues such as the development of: 1) principles of formal analysis, as presented above, that could be used by analysts to conduct studies and by decisionmakers to evaluate and use studies; 2) "array" or nonaggregated methods of presenting and considering costs and benefits; 3) measurement indexes that attempt to capture and weigh divergent variables; and 4) analytical approaches to various categories of decisions in health care, for example, the use of diagnostic technology, or assessment of new versus established technologies. This research could be coordinated with the studies that would be conducted under *Option 8*. Those studies, in order to help determine the potential usefulness of CEA/CBA, should be undertaken or at least designed and evaluated in collaboration with agencies making policy decisions relating to medical technology. Option 8 cannot be merely an academic exercise; it cannot be done in a policy vacuum. If it is, few relevant lessons about CEA/CBA will result. Further, because the number of technologies that could be

studied is extremely large, the setting of priorities for selecting those to be analyzed is of critical importance.

Option 9 does not mean the collection of additional data. OTA believes that such collection should be postponed until much more thought has gone into a strategy that specifies the most useful kinds and forms of information needed to conduct analyses of costs and benefits. The usefulness of CEA/CBA is critically dependent on the quality of the data available. Currently, the state of many types of data, especially data on efficacy and safety of medical technology, is inadequate. Much work has already gone into examining the state of health data systems and content. The strategy envisioned by Option 9 would build upon the existing studies, but would require consideration of the specific needs of CEA/CBA analysts and decisionmakers.

The need for some method of comparing the effects of health care activities with the resources consumed will remain critical to policymakers. Thus, the potential of CEA/CBA or some derivation should be explored fully. This may require, or at least benefit from, limited experiments on the actual formal application of CEA/CBA in program decisionmaking. Three possible areas for the demonstration projects of *Option 10* may be medicare's reimbursement coverage decisions, the resource allocation activities of health planning agencies, and the review activities of the PSROs. Option 10 recognizes that the methods of CEA/CBA may improve, that data inadequacies may be lessened, and that methods of assuring the appropriate consideration of CEA/CBA results (in relation to other variables of the decision) may be developed. Thus, today's judgments of the role and usefulness of CEA/CBA may need modification later. In fact, demonstration projects may help to advance the usefulness of CEA/CBA by contributing to advances in methods, data, and so on.

DESCRIPTION OF BACKGROUND PAPERS

This document summarizes the major report of the project on The Implications of Cost-Effectiveness Analysis of Medical Technology. In addition to that full report, several background papers were prepared and are available separately. The findings and options summarized above are in large part derived from the information generated by the background efforts.

Background Paper #1: Methodological Issues and Literature Review, includes an indepth examination of the decisionmaking con-

text and methodology discussions presented in this summary. A critique of the literature, a bibliography of approximately 600 items, and abstracts of over 70 studies and other articles are also included.

In order to investigate the applicability of formal techniques for assessing the costs and benefits of medical technologies, 19 case studies were prepared. These are available individually, and a collected volume, *Background Paper #2: Case Studies of Medical Technologies*, contains 17 of the cases. Some of the cases represent formal CEAs (e.g., bone marrow transplants), while others represent net cost analyses (e.g., selected respiratory therapies). Other cases illustrate various issues such as the difficulty of conducting CEA in the absence of adequate efficacy and safety information (e.g., breast cancer surgery), or the role and impact of formal analysis on policymaking (e.g., end-stage renal disease interventions). The 17 case studies in the background paper are:

Screening for cervical cancer
Artificial heart
End-stage renal disease interventions
Elective hysterectomy
CT scanning
Selected respiratory therapies
Periodontal disease
Cardiac radionuclide imaging
Automated chemistry analyzers
Upper gastrointestinal endoscopy
Breast cancer surgery
Screening for colon cancer
Neonatal intensive care units
Nurse practitioners
Orthopedic joint prosthetic implants
Cimetidine and peptic ulcer disease
Bone marrow transplants

The 18th case study is published separately as *Background Paper #3: The Efficacy and Cost-Effectiveness of Psychotherapy*. That study assesses methodological and substantive issues relating to the scope of psychotherapy, the evaluation of psychotherapeutic efficacy, and the applicability of CEA and CBA in assessing psychotherapy. *Background Paper #5: Evaluation of Four Common X-Ray Procedures* contains the 19th case study.

Background Paper #4: The Management of Health Care Technology in Ten Countries is an analysis of the programs and methods, including cost-effectiveness and cost-benefit techniques, that nine industrialized nations use to control the use of medical technologies. These countries' experience in managing medical technologies is compared to the experience of the United States. The nine countries are: the United Kingdom, Canada, Australia, Japan, France, West Germany, the Netherlands, Iceland, and Sweden.

Note: Copies of the full report and the five Background Papers (when they become available) can be purchased from the Superintendent of Documents, U.S. Government Printing Office, Washington, D.C. 20402.

Studies in Progress (as of August 1980)

Alternative Energy Futures
Solar Power Satellite Systems
Global Energy Trends—Global Oil
Synthetic Fuels for Transportation
Decentralized Electric Energy Generating Systems
An Analysis of Nuclear Powerplant Standardization Problems
An Assessment of Development and Production Potential of Federal Coal Leases
An Assessment of Nonnuclear Industrial Hazardous Waste
Impact of Technology on Competitiveness of U.S. Electronics Industry
U.S. Industrial Competitiveness: A Comparison of Steel, Electronics, and Automobiles
Technology and Soviet Energy Availability
MX Missile Basing
U.S. Food and Agricultural Research
Impact of Technology on Productivity of the Land
Technologies for Determining Cancer Risks From the Environment
Evaluation of Veterans Administration Agent Orange Protocol
Technologies for the Handicapped
Strategies for Medical Technology Assessment
Impacts of Applied Genetics
Technology and World Population
An Assessment of Technology for Local Development
National Laboratories—Oversight, Legislative, and Authorization Issues
Technological Innovation and Health, Safety, and Environmental Regulations
An Assessment of High-Level Radioactive Waste Management and Disposal
Freshwater Resources Management, Planning, and Policy: An Assessment of Models and Predictive Methods
Ocean Research Technology
Assessment of the Societal Impact of National Information Systems
Societal Impact of Telecommunications Technology
Space Policy and Applications
Impacts of the 1979 World Administrative Radio Conference
Impact of Advanced Air Transport Technology
Airport and Air Traffic Control System
Automotive Fuel Efficiency and Alternative Energy Sources

General Information

Information on the operation of OTA, the nature and status of ongoing assessments, or a list of available publications may be obtained by writing or calling:

Public Communications Office
Office of Technology Assessment
U.S. Congress
Washington, D.C. 20510
(202) 224-0885

Publications Available

OTA Annual Report.—Details OTA's activities and summarizes reports published during the preceeding year.

List of Publications.—Catalogs by subject area all of OTA's published reports with instructions on how to order them.

Publication Briefs.—Summarize reports and findings of assessments.

Press Releases.—Announce publication of reports, staff appointments, and other newsworthy activities.

OTA Brochure.—"What It Is, What It Does, How It Works."

Ongoing Assessments.—Contains brief descriptions of assessments presently underway with estimated dates of completion.

Contacts Within OTA

(OTA offices are located at 600 Pennsylvania Avenue, S.E., Washington, D.C.)

Office of the Director	224-3695
Office of the Deputy Director	224-3695
Congressional and Institutional Relations	224-9241
Energy, Materials, and International Security Division	224-0732
Health and Life Sciences Division	224-1047
Science, Information, and Transportation Division	224-0732
Administration Office	224-8712
Personnel Office	224-8713
Publications	224-8996

U.S. GOVERNMENT PRINTING OFFICE : 1980 O - 66-259

CONGRESS OF THE UNITED STATES
OFFICE OF TECHNOLOGY ASSESSMENT
WASHINGTON, D.C. 20510

OTA-H-125